HIV/AIDS Fact Sheet

CIRM funds many projects seeking to better understand HIV/AIDS and to translate those discoveries into new therapies.

Description

HIV, or the human immunodeficiency virus, is a virus that infects cells of the immune system, undermining the body's ability to fight infection and disease. Eventually infection can lead to symptoms of AIDS (Acquired Immune Deficiency Syndrome), which includes susceptibility to infections, cancers and other diseases, and eventually causes death. According to the CDC, more than 1.1 million people in the U.S. are infected with HIV at this moment.

Stem cell approaches to treating people with HIV primarily involve replacing the person's immune system with one that the virus can't infect. Hope that this approach could work were boosted in late 2010 when scientists reported that Timothy Ray Brown, also known as the "Berlin Patient", had effectively had his HIV "cured". As part of a treatment for leukemia, Brown had received a bone marrow transplant that came from a donor whose cells were resistant to HIV infection.

The person who donated the bone marrow had a genetic mutation in a gene called CCR5, which makes a protein that is required for HIV to enter cells. Without CCR5, HIV wasn't able to infect these replacement immune cells and Brown has been able to go off his medications.

The problem is that there aren't enough people with naturally occurring CCR5 mutations to serve as bone marrow donors for all HIV patients. Instead, scientists are hoping to create CCR5 mutations. They first plan to remove the blood-forming stem cells in a person's bone marrow and mutate the CCR5 gene. The idea is that those genetically altered cells would then repopulate the person's blood system with one that lacks CCR5 and that HIV won't be able to infect.

Clinical Stage Programs

City of Hope

The City of Hope team plans to mutate the CCR5 gene using a technology called a zinc finger nuclease, which is essentially a pair of molecular scissors developed by Sangamo Biosciences that snips an exact spot on the CCR5 gene. Early evidence in animals suggests that when those cells are reintroduced, they create an immune system that HIV can't infect. The team has begun a clinical trial with the procedure.

- Read a summary of this project

Calimmune

The Calimmune team is using a method called RNA interference to block the CCR5 gene from generating a protein. A blood system generated from these cells will lack CCR5 and block HIV infection. The team has begun a clinical trial with the procedure.

- Read a summary of this project

University of California, Davis

The team at is taking a patient's blood forming stem cells and inserting 3 anti-HIV genes into them and then returning them to the individual. The anti-HIV genes are then passed on to all new immune system cells and make them resistant to HIV. Because AIDS-related lymphoma is linked to the constant immune cell stimulation caused by HIV infection, getting rid of the virus should prevent return of the cancer.

- Read a summary of this project

Jeff Sheehy, HIV/AIDS patient advocate member of the CIRM Governing Board, and John Zaia, leader of the City of Hope CIRM HIV Disease Team, discuss stem cell transplant strategies for the treatment of HIV/AIDS.

CIRM Grants Targeting HIV/AIDS

Researcher name	Institution	Grant Title	Grant Type	Approved funds
Paula Cannon	University of Southern California	Site-specific gene editing in hematopoietic stem cells as an anti-HIV therapy	Tools and Technologies III	$1,499,400
Mehrdad Abedi	University of California, Davis	Stem Cell Gene Therapy for HIV Mediated by Lentivector Transduced, Pre-selected CD34+ Cells in AIDS lymphoma patients	Clinical Trial Stage Projects	$8,414,265
David Baltimore	California Institute of Technology	Immunotherapy for HIV infection using engineered hematopoietic stem/progenitor cells	Quest - Discovery Stage Research Projects	$1,586,934
David DiGiusto	City of Hope	Development of RNA-based approaches to stem cell gene therapy for HIV	Early Translational II	$3,097,160
Irvin Chen	University of California, Los Angeles	Stem Cells: A New Avenue of HIV Research and New Approaches to HIV Treatment	Conference	$24,456
John Zaia	City of Hope	ZINC FINGER NUCLEASE-BASED STEM CELL THERAPY FOR AIDS	Disease Team Research I	$14,536,969

Irvin Chen	University of California, Los Angeles	HPSC based therapy for HIV disease using RNAi to CCR5.	Disease Team Research I	$9,905,604	
Irvin Chen	University of California, Los Angeles	Genetic modification of the human genome to resist HIV-1 infection and/or disease progression	SEED Grant	$616,800	
Zack Jerome	University of California, Los Angeles	Human Embryonic Stem Cell Therapeutic Strategies to Target HIV Disease	Comprehensive Grant	$2,401,903	
Geoff Symonds	Calimmune, Inc.	GENE-MODIFIED HEMATOPOIETIC STEM/PROGENITOR CELL BASED THERAPY FOR HIV DISEASE	Disease Team Research I	$8,278,722	
Mark Anderson	University of California, San Francisco	Stem cell differentiation to thymic epithelium for inducing tolerance to stem cells	Transplantation Immunology	$1,314,089	
Mehrdad Abedi	University of California, Davis	Stem Cell Gene Therapy for HIV in AIDS Lymphoma Patients	Disease Team Therapy Planning I	$66,880	
Zack Jerome	University of California, Los Angeles	Stem Cell Programming With Chimeric Antigen Receptors to Eradicate HIV Infection	Early Translational IV	$4,925,166	
Irvin Chen	University of California, Los Angeles	Development of a humanized mouse model for testing anti-HIV HSPC gene therapy strategies in HIV-1 infected mice.	Early Translational from Disease Team Conversion	$1,505,000	
Mark Anderson	University of California, San Francisco	Generation of a functional thymus to induce immune tolerance to stem cell derivatives	Basic Biology V	$1,191,000	
John Zaia	City of Hope	A Phase I, Open-Label Study To Assess The Safety, Feasibility and Engraftment of Zinc Finger Nucleases (ZFN) CCR5 Modified Autologous CD34+ Hematopoietic Stem/Progenitor Cells (SB-728MR-HSPC) with Escalating Doses of Busulfan In HIV-1 (R5) Infected Subjec	Strategic Partnership III Track A	$5,583,438	
John Zaia	City of Hope	The Innovation-Alpha Clinic for Cellular Therapies (I-ACT) – A Program for the Development and Delivery of Innovative Cell-based Treatments and Cures for Life-threatening Diseases.	Alpha Stem Cell Clinics	$8,000,000	
					Total: $72,947,786.00

CIRM HIV/AIDS Videos

 **Is a HIV Cure Possible? A Lecture Series on HIV Cure Research**	 **Is a HIV Cure Possible? A Panel Discussion on HIV Cure Research**	 **HIV/AIDS Stem Cell Clinical Trial: Introduction by Jeff Sheehy**	 **HIV/AIDS Stem Cell Clinical Trial: Louis Breton, CEO, Calimmune**
 **HIV/AIDS Stem Cell Clinical Trial: Geoff Symonds, Calimmune**	 **John Zaia, City of Hope - CIRM Stem Cell #SciencePitch**	 **HIV/AIDS: Advancing Stem Cell Therapies: 2011 CIRM Grantee Meeting**	 **Progress and Promise in HIV/AIDS**
 **Spotlight on Disease Team Awards - HIV/AIDS: Jeff Sheehy**	 **Spotlight on Disease Team Awards - HIV/AIDS: John Zaia**	 **Spotlight on Disease Team Awards - HIV/AIDS: Loring Leeds**	 **Biotech Perspectives for Stem Cell Research: Don Francis**
 **HIV and Stem Cells: Warner Greene - CIRM Science Writer's Seminar**			

Resources

- CIRM Blogs on HIV/AIDS Research
- CDC: Information about HIV/AIDS
- NIH: AIDS Information
- AIDS Policy Project
- The Body
- Project Inform
- HIVinSite
- Find a clinical trial near you: NIH Clinical Trials database
- The Foundation for AIDS Research
- San Francisco AIDS Foundation
- National AIDS Fund
- AIDS Research Institute, UCSF
- Family Caregiver Alliance
- National Family Caregivers Association

Find Out More:
Stem Cell FAQ | Stem Cell Videos | What We Fund

Source URL: https://www.cirm.ca.gov/our-progress/disease-information/hivaids-fact-sheet